

## PHP5

## INNOVATIVE METHODS FOR STAKEHOLDER ENGAGEMENT: AN ENVIRONMENTAL SCAN

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**OBJECTIVES:** Involving stakeholders in research helps ensure that research is relevant and responsive to stakeholder needs while improving the quality of the research. This research was conducted as part of AHRQ's Community Forum project which was charged with identifying emerging strategies and state of the art methods for engaging stakeholders. **METHODS:** We developed a conceptual framework for stakeholder engagement that informed this literature scan and assisted in organizing findings. The scan included a search of peer-reviewed literature, using academic databases as well as an Internet search of grey literature and Web sites. Our final review included 23 peer-reviewed articles, 15 grey literature documents, and 43 Web sites related to stakeholder engagement. To supplement our scan, we conducted 11 interviews with individuals experienced in the field of stakeholder engagement. Our technical experts also suggested organizations and Web sites to explore in our literature review. **RESULTS:** We identified many promising methods of involving stakeholders in research. One example is using online collaborative platforms to enable interactions between an organization and its stakeholders through a virtual space. These allow users to share information, work together, and provide feedback to stakeholders about how their input is being used. Other examples include product development challenges, utilizing existing online communities, and grassroots community organizing. Selected recommended practices from our review include gaining trust of stakeholders before involvement and maintaining throughout; selecting stakeholders for whom the decision or research has important consequences; and educating stakeholders on their roles, their responsibilities, and the topics being discussed. A limitation to our findings is that there are limited evaluation data measuring outcome effectiveness of these methods. **CONCLUSIONS:** Stakeholder involvement is critical to health care research. This presentation will highlight selected effective and creative approaches to stakeholder engagement.

## HEALTH CARE USE &amp; POLICY STUDIES – Disease Management

## PHP6

## PROFILE OF CARE FOR SEXUALLY TRANSMITTED INFECTIONS (STIS) IN HEALTH CENTERS IN BORDER AREAS OF CENTRAL AMERICA

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**OBJECTIVES:** To analyze the profile of care for sexually transmitted infections (STIs) in health centers in border areas of Central America. **METHODS:** A cross-sectional study in a sample of 3357 people received medical care for some STIs in 13 health centers in border areas of Central America, during 2007-2010. Doctors were trained and supplied medicines, condoms and HIV testing (basic package of health care). The sample of users was characterized according to sociodemographic variables and the associated factors with the probability of receiving the basic package of care for STIs were analyzed using additive probit regression models. **RESULTS:** Of the total users, 66% had 25 to 59 years old, and 93% of users were women. The most frequently diagnosed STI were Candidiasis, Bacterial Vaginosis, Trichomoniasis, Gonorrhea and Syphilis. 60% of prescriptions for these STIs were adhered to the international recommendations. 53.1 % of users received only medicines, 5.8 % received medicines plus HIV testing, 30.8 % received medicines plus condoms and only 10% of them received the complete basic package. The likelihood of receiving was lower in women. **CONCLUSIONS:** It is not enough to increase service delivery capacity to change care practices. These are deeply rooted in the sociocultural context related notions and cultural biases. Highlights gendered medical practices that adversely affect the profile of care.

## PHP7

## RECENT DEVELOPMENTS IN POMPE DISEASE THERAPY

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**OBJECTIVES:** Pompe disease is a rare disease that affects between 5,000 and 10,000 people worldwide. It is an inherited metabolic myopathy caused by deficiency of acid alpha-glucosidase (GAA) enzyme in lysosomal cells. The purpose of this study was to review and compare recent developments in Pompe disease therapy. **METHODS:** Using PubMed and other Internet-based search engines, we reviewed Pompe disease epidemiology and recent new drug development, and compared two newly-approved therapies in terms of their indications, efficacy, and safety profiles. **RESULTS:** The incidence of Pompe disease varies among different populations, ranging from 0.0032% in African Americans to 0.002% for the Chinese. There are two forms of Pompe disease: infantile-onset and late-onset. The survival rate to age one is about 25.7% for those with the infantile-onset disease. Two new drugs are both enzyme replacement therapies (ERTs), including Myozyme® (alglucosidase alfa, rhGAA) and Lumizyme® (alglucosidase alfa). They were approved as orphan drugs by the U.S. Food and Drug Administration in 2006 and 2010, respectively. Both are biologics that have been marketed around the world. While Myozyme® is indicated for infantile-onset, Lumizyme® is for children 8 years old or older. Both drugs improve the survival rate, but there remains a need to monitor risk of heart and lung failure among those on treatment. **CONCLUSIONS:** Although ERT is an approved effective treatment for Pompe disease, gene therapy is under development in order to correct sequence coding gene for the deficient enzyme into cells.

## HEALTH CARE USE &amp; POLICY STUDIES – Drug/Device/Diagnostic Use &amp; Policy

## PHP8

## A REVIEW OF ECONOMIC EVALUATIONS ON NEW DRUGS PRICED BY THE COST CALCULATION METHOD IN JAPAN. CAN JAPAN GO BEYOND THE CONVENTIONAL COST-BASED PRICING?

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**OBJECTIVES:** To explore the data sources used in economic evaluations performed on new drugs, and to propose an improved data infrastructure in order to deal with future value-based decision making in health care policy in Japan. **METHODS:** We systematically reviewed economic evaluation studies of the new drugs which were launched in Japan between April 2006 and March 2011, and which have been priced by the cost calculation method, one of two types of pricing for new drugs in Japan. The "Ichushi" and Pubmed databases were used to find the published articles. **RESULTS:** A total of 198 drugs were priced under cost calculation methods in the last 5 years in Japan. Fourteen published articles (9 drugs) were found: 5 CUA and 10 CEAs (including one that was both CUA and CEA). In all studies, several data from different sources were incorporated. Cost data were estimated by using standard treatment protocols and national price lists for drugs and medical services, or obtained from a limited number of claims data. Efficacy data were obtained from RCTs or clinical trial data mostly conducted in Japan. In 4 out of the 5 CUAs, utility data were used from other studies conducted on non-Japanese samples, and 2 CUAs did not consider transferability issues. Other data, such as epidemiological data, were adopted from overseas as well as Japanese statistics; usually mortality rates were from Japanese statistics, while incident rates were from overseas. **CONCLUSIONS:** In order to increase quality and efficiency to conduct economic evaluations on new drugs, three steps need to be taken in the data environment: increased accessibility to large cost databases such as the national claims database; establish an epidemiological database; and collect and accumulate utility data in Japanese samples.

## PHP9

## CONSENSUS-BASED POLICY SOLUTIONS FOR MEDICINES ADHERENCE FOR EUROPE: DEVELOPMENT AND EVALUATION

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**OBJECTIVES:** Despite much research to understand patient nonadherence, clear research-based evidence of how to reduce non-adherence remains elusive. This paper describes two related studies conducted as part of the FP7 European Commission funded Ascertaining Barriers to Compliance (ABC) project. We sought to develop consensus-based policy solutions to address medicines adherence and benchmark current implementation activity for those policy solutions. **METHODS:** Delphi study: A 4-round Delphi study was conducted with 49 participants from 14 countries, representative of a range of stakeholder groups. Participants engaged in the study remotely, anonymously and electronically. Participants responded to open questions about the causes, consequences and solutions to medication non adherence. Subsequent rounds refined responses, and sought ratings of the relative importance, operational and political feasibility of each potential solution to medication nonadherence. Feedback of responses was provided to participants after each round. A consensus meeting was held to discuss and further refine the proposed policy solutions. National self-assessment study: An online survey and follow-up telephone interview were conducted based on policy solutions resulting from the consensus study. National medicines leads within the Ministries of Health for the 27 European Union member states were invited to participate. Participants rated the extent and appropriateness of implementation of each policy solution in their country. **RESULTS AND CONCLUSIONS:** Twenty-five policy solutions were prioritised based on composite scores for importance, operational and political feasibility. Prioritised policy solutions focused on interventions for patients, training for healthcare professionals, and actions to support patient-clinician partnership. Few solutions concerned actions by governments, health care payers, or interventions at the system level. Implementation varied across nations. Some European nations had not identified medication adherence as a priority and tended to be concerned with developing more general clinical governance arrangements. Some European nations had more systematic, co-ordinated approaches to implementing policy solutions to address medication nonadherence.

## PHP10

## AUSTRALIA'S RULE OF RESCUE AND THE LIFE SAVING DRUGS PROGRAMME: SAME-SAME BUT DIFFERENT?

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**OBJECTIVES:** Medicines that are considered to be clinically effective but not cost-effective by the Pharmaceutical Benefits Advisory Committee (PBAC) may be reimbursed in Australia through two pathways: by meeting the 'rule of rescue' (RoR) criteria to be listed on the Pharmaceutical Benefits Scheme, or being included on the life-saving drugs programme (LSDP). The criteria for the RoR and LSDP share some similarities, but the LSDP criteria are less subjective and more restrictive. We sought to analyse and compare outcomes for submissions that were considered under the RoR and LSDP from 2008 to 2011. **METHODS:** Public Summary Documents of PBAC recommendations for 2008-2011 were reviewed to identify submissions that were considered for reimbursement citing the RoR or requested listing on the LSDP. Outcomes of the applications were analysed and Payer interviews were un-